

Union Calendar No. 410

103D CONGRESS
2D SESSION

H. R. 4865

[Report No. 103-746]

A BILL

To amend the Federal Food, Drug, and Cosmetic Act, the Public Health Service Act, and the Orphan Drug Act to revise the provisions of such Acts relating to orphan drugs.

SEPTEMBER 26, 1994

Reported with an amendment, committed to the Committee of the Whole House on the State of the Union, and ordered to be printed

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IN THE HOUSE OF REPRESENTATIVES

AUGUST 1, 1994

Mr. WAXMAN (for himself and Mr. STUDDS) introduced the following bill;
which was referred to the Committee on Energy and Commerce

SEPTEMBER 26, 1994

Reported with an amendment, committed to the Committee of the Whole
House on the State of the Union, and ordered to be printed

[Strike out all after the enacting clause and insert the part printed in italic]

[For text of introduced bill, see copy of bill as introduced on August 1, 1994]

A BILL

To amend the Federal Food, Drug, and Cosmetic Act, the Public Health Service Act, and the Orphan Drug Act to revise the provisions of such Acts relating to orphan drugs.

1 *Be it enacted by the Senate and House of Representa-*
2 *tives of the United States of America in Congress assembled,*

1 **SECTION 1. SHORT TITLE AND REFERENCE.**

2 (a) *SHORT TITLE.*—This Act may be cited as the
3 “Orphan Drug Act Amendments of 1994”.

4 (b) *REFERENCE.*—Whenever in this Act (other than
5 sections 5 and 6) an amendment or repeal is expressed in
6 terms of an amendment to, or repeal of, a section or other
7 provision, the reference shall be considered to be made to
8 a section or other provision of the Federal Food, Drug, and
9 Cosmetic Act.

10 **SEC. 2. PERIOD OF EXCLUSIVITY.**

11 (a) *INITIAL PERIOD.*—Subsection (a) of section 527
12 (21 U.S.C. 360cc) is amended—

13 (1) by inserting “(1)” after “(a)”,

14 (2) by redesignating paragraphs (1), (2), and (3)
15 as subparagraphs (A), (B), and (C), respectively,

16 (3) by striking “seven years” and inserting “4
17 years”, and

18 (4) by striking “505(c)(2)” and inserting
19 “505(c)(1)(B)”.

20 (b) *ADDITIONAL PERIOD.*—Subsection (a) of such sec-
21 tion 527 (21 U.S.C. 360cc) (as amended by subsection (a))
22 is amended by adding at the end the following:

23 “(2) The holder of the approved application, certifi-
24 cation, or license of a drug to which the 4-year period of
25 exclusivity applies under paragraph (1) may, after the ex-
26 piration of 3½ years of such period but not later than 90

1 *days before the expiration of such period, apply to the Sec-*
2 *retary for a 3-year extension of such period. Such an appli-*
3 *cation shall contain such information as the Secretary de-*
4 *termines is necessary to evaluate such application.*

5 *“(3) The Secretary shall approve an application sub-*
6 *mitted under paragraph (2) if the applicant—*

7 *“(A) demonstrates that the drug has a limited*
8 *commercial potential, as determined under regula-*
9 *tions of the Secretary, on the basis of—*

10 *“(i) total sales revenue for such drug during*
11 *the 4-year period of exclusivity under paragraph*
12 *(1), or*

13 *“(ii) factors other than total sales revenue*
14 *identified by the Secretary, and*

15 *“(B) makes such demonstration on the basis of*
16 *the regulations of the Secretary referred to in sub-*
17 *paragraph (A) which were in effect—*

18 *“(i) on the date—*

19 *“(I) such drug received its designation*
20 *under section 526(a)(1), or*

21 *“(II) such applicant applied for an ex-*
22 *emption for such drug under section 505(i)*
23 *or clause (3) of section 507(d),*

24 *whichever first occurs, or*

1 “(ii) if the date under clause (i) occurred
2 before the date such regulations were in effect, on
3 the date such regulations were in effect.”.

4 (c) *CONFORMING AMENDMENTS.*—Section 527(b) (21
5 *U.S.C. 360cc(b)*) is amended—

6 (1) by striking “during the seven-year period”
7 and inserting “during the applicable period of exclu-
8 sivity under subsection (a)”,

9 (2) in paragraph (2), by striking “such seven
10 year period” and inserting “the applicable period of
11 exclusivity under subsection (a)”,

12 (3) by striking “507,,” and inserting “507,”, and

13 (4) in paragraph (1), by striking “The Sec-
14 retary” and inserting “the Secretary”.

15 (d) *EFFECTIVE DATE.*—The amendments made by
16 subsections (a) and (b) shall not apply to a drug—

17 (1) for which an application under section 505
18 or 507 of the Federal Food, Drug, and Cosmetic Act
19 or section 351 of the Public Health Service Act was
20 submitted or approved before March 1, 1994, or

21 (2) for which an exemption under section 505(i)
22 or clause (3) of section 507(d) of the Federal Food,
23 Drug, and Cosmetic Act was in effect before March 1,
24 1994, for which human clinical trials were actively
25 being conducted before such date, and for which an

1 *application for designation under section 526 of such*
2 *Act was submitted before the date of enactment of the*
3 *Orphan Drug Act Amendments of 1994.*

4 *The 7-year period of exclusivity provided by section 527(a)*
5 *of the Federal Food, Drug, and Cosmetic Act before the date*
6 *of the enactment of this Act shall, after such date, apply*
7 *to a drug described in paragraph (1) or (2).*

8 *(e) REGULATIONS.—The Secretary shall issue final*
9 *regulations to implement paragraphs (2) and (3) of section*
10 *527(a) of the Federal Food, Drug, and Cosmetic Act (21*
11 *U.S.C. 360cc) (as added by subsection (b)) not later than*
12 *6 months after the date of the enactment of this Act.*

13 **SEC. 3. DESIGNATIONS.**

14 *(a) IN GENERAL.—Section 526(a)(2) (21 U.S.C.*
15 *360bb(a)(2)) is amended to read as follows:*

16 *“(2) For purposes of paragraph (1), the term ‘rare dis-*
17 *ease or condition’ means any disease or condition which—*

18 *“(A) affects fewer than 200,000 persons in the*
19 *United States determined on the basis of—*

20 *“(i) the facts and circumstances as of the*
21 *date the request for designation of the drug*
22 *under this subsection is made, and*

23 *“(ii) projections as to the number of persons*
24 *who will be affected by the disease or condition*

1 on a date which is 3 years from date such re-
2 quest was made, or

3 “(B) affects more than 200,000 persons in the
4 United States and for which there is no reasonable ex-
5 pectation that the cost of developing and making
6 available in the United States a drug for such disease
7 or condition will be recovered from sales in the Unit-
8 ed States of such drug.”.

9 (b) *EXCLUSIVITY*.—Section 527(b) (21 U.S.C.
10 360cc(b)) is amended—

11 (1) by striking “or” at the end of paragraph (1),

12 (2) by striking the period at the end of para-
13 graph (2) and inserting a semicolon, and

14 (3) by adding at the end the following:

15 “(3) such drug was designated under section
16 526(a)(1) for a rare disease or condition described in
17 paragraph (2) of section 526(a) and, after such des-
18 ignation, it is determined that such disease or condi-
19 tion affects more than 200,000 persons in the United
20 States; or”.

21 **SEC. 4. SIMULTANEOUS DEVELOPMENT.**

22 (a) *IN GENERAL*.—Section 527(b) (21 U.S.C.
23 360cc(b)), as amended by section 3(b), is amended by in-
24 serting “(1)” after “(b)”, by redesignating paragraphs (1),
25 (2), and (3) as subparagraphs (A), (B), and (C), respec-

1 tively, by striking “for a person who is not” and by insert-
2 ing “for an applicant who is not”, and by adding at the
3 end the following:

4 “(D) the Secretary finds, after providing the
5 holder, such applicant, and any other interested per-
6 son an opportunity to present their views, that the
7 drugs of the holder and such applicant were developed
8 simultaneously.

9 The Secretary shall make a decision on a request for a find-
10 ing under subparagraph (D) not later than 60 days after
11 the filing of the request.

12 “(2) For purposes of paragraph (1)(D), drugs of a
13 holder and an applicant shall be considered to be developed
14 simultaneously only if—

15 “(A) the applicant requested that its drug be des-
16 ignated under section 526 no later than 6 months
17 after publication of the designation under section
18 526(c) of the holder’s drug,

19 “(B) the applicant initiated the human clinical
20 trials that the applicant relied on in its application
21 for such approval, certification, or license not more
22 than 12 months after the date the holder initiated the
23 human clinical trials that the holder relied on in its
24 application for such approval, certification, or
25 license, and

1 “(C) the applicant submitted such application,
2 including the reports of the clinical and animal stud-
3 ies necessary for approval, certification, or license,
4 not more than 12 months after the holder submitted
5 its application, including such reports, for such
6 action.

7 “(3) Paragraph (1)(D) does not apply to a drug—

8 “(A) for which an application under section 505
9 or 507 or section 351 of the Public Health Service Act
10 was submitted or approved before March 1, 1994, or

11 “(B) for which an exemption under section
12 505(i) or clause (3) of section 507(d) was in effect be-
13 fore March 1, 1994, for which human clinical trials
14 were actively being conducted before such date, and
15 for which an application for designation under sec-
16 tion 526 was submitted before the date of enactment
17 of the Orphan Drug Act Amendments of 1994.”.

18 (b) PUBLICATION.—Section 526(c) (21 U.S.C.
19 360bb(c)) is amended—

20 (1) by inserting “for a rare disease or condition”
21 after “(a)”, and

22 (2) by striking out “shall be made available to
23 the public” and inserting in lieu thereof “shall be
24 promptly published in the Federal Register and other-
25 wise made available to the public in a manner de-

1 *signed to notify persons who have such disease or con-*
2 *dition”.*

3 **SEC. 5. OFFICE FOR ORPHAN DISEASES AND CONDITIONS.**

4 *Section 227 of the Public Health Service Act (42*
5 *U.S.C. 236) is amended—*

6 *(1) by amending subsection (a) to read as*
7 *follows:*

8 *“(a) There is established in the Department of Health*
9 *and Human Services an Office for Orphan Diseases and*
10 *Conditions. Such Office shall be established at a level within*
11 *the Department with sufficient authority to assure full im-*
12 *plementation of the functions and responsibilities estab-*
13 *lished by this section.”,*

14 *(2) by striking “Board” each place the term ap-*
15 *pears and inserting “Office”,*

16 *(3) by striking “drugs and devices” in subsection*
17 *(b) and inserting “drugs, devices, and medical foods”,*

18 *(4) by inserting “of chapter V” after “subchapter*
19 *B” in subsection (c)(1)(A),*

20 *(5) by adding at the end the following new sub-*
21 *section:*

22 *“(f)(1) There is established in the Office an advisory*
23 *committee to advise the Office in carrying out the functions*
24 *of the Office under this section.*

1 “(2) *The advisory committee shall be comprised of 11*
2 *members appointed by the Secretary, in consultation with*
3 *the Office and the Commissioner of Food and Drugs, from*
4 *persons knowledgeable about rare diseases and conditions,*
5 *including—*

6 “(A) *5 representatives of organizations of persons*
7 *with rare diseases or conditions;*

8 “(B) *3 research scientists; and*

9 “(C) *3 representatives of health-related compa-*
10 *nies.*

11 “(3) *The Secretary shall also appoint, as liaisons to*
12 *the advisory committee, individuals from the Food and*
13 *Drug Administration, the National Institutes of Health,*
14 *and other appropriate Federal agencies.*

15 “(4) *Vacancies occurring in the membership of the ad-*
16 *visory committee shall be filled in the same manner as the*
17 *original appointment for the position being vacated. Vacan-*
18 *cies shall not affect the power of the remaining members*
19 *to execute the duties of the advisory committee.*

20 “(5) *Members of the advisory committee, and liaisons*
21 *to the advisory committee, shall not be compensated, but*
22 *shall receive travel expenses, including per diem in lieu of*
23 *subsistence, at rates authorized for employees of agencies*
24 *under subchapter 1 of chapter 57 of title 5, United States*
25 *Code, for each day the member or liaison is engaged in the*

1 *performance of duties away from the home or regular place*
2 *of business of the member or liaison.*

3 “(6) Notwithstanding section 1342 of title 31, United
4 States Code, the advisory committee may accept the vol-
5 untary services provided by a member of the advisory com-
6 mittee or a liaison to the advisory committee.”, and

7 (6) by amending the section heading to read as
8 follows: “OFFICE FOR ORPHAN DISEASES AND CONDI-
9 TIONS”.

10 **SEC. 6. AUTHORIZATION FOR ORPHAN DRUG ACT.**

11 Section 5(c) of the Orphan Drug Act (21 U.S.C.
12 360ee(c)) is amended by striking “\$10,000,000” and all
13 that follows and inserting “\$20,000,000 for fiscal year
14 1995, \$25,000,000 for fiscal year 1996, and \$30,000,000 for
15 fiscal year 1997.”.